CIRM Funded Clinical Trials

# A Phase I/II, Non Randomized, Multicenter, Open-Label Study of G1XCGD (Lentiviral Vector Transduced CD34+ Cells) in Patients With X-Linked Chronic Granulomatous Disease

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| **Disease Area:** | X-linked Chronic Granulomatous Disease |
| **Investigator:** | Donald Kohn |
| **Institution:** | University of California, Los Angeles |
| **CIRM Grant:** | CLIN2-08231 |
| **Award Value:** | $7,083,364 |
| **Trial Sponsor:** | University of California, Los Angeles |
| **Trial Stage:** | Phase 1/2 |
| **Trial Status:** | Suspended |
| **Targeted Enrollment:** | 10 |
| **ClinicalTrials.gov ID:** | NCT02234934 |

Donald Kohn

**Details:**
X-linked Chronic Granulomatous Disease (X-CGD) is a rare immune disorder that prevents white blood cells from killing foreign invaders. This results in severe, recurrent infections that can impact quality and length of a patient's life. X-CGD is usually diagnosed before age 5, but without treatment, children die before age 10. A team at UCLA is using the patient's own genetically modified blood stem cells to create a new blood supply and a healthy immune system, with the aim of curing patients with this disease.

**Design:**
X-linked Chronic Granulomatous Disease.

**Goal:**
Primary: Safety and Efficacy. Secondary: Restoration of immune function

**Updates:**
Enrolling. Early evidence of clinical efficacy.

Contact Trial Sponsor

**Source URL:** https://www.cirm.ca.gov/clinical-trial/phase-iii-non-randomized-multicenter-open-label-study-g1xcgd-lentiviral-vector